
Stem cells for neuroprotection of photoreceptors in retinitis pigmentosa

Grant Award Details

Stem cells for neuroprotection of photoreceptors in retinitis pigmentosa

Grant Type: Disease Team Planning

Grant Number: DT1-00690

Investigator:

Name: Henry Klassen

Institution: University of California, Irvine

Type: PI

Award Value: \$23,537

Status: Closed

Grant Application Details

Application Title: Stem cells for neuroprotection of photoreceptors in retinitis pigmentosa

Public Abstract: The targeted disease is retinitis pigmentosa (RP), a severe form of blindness that runs in families. This disease is not common, yet represents an attractive near term target for stem cell therapy for a number of reasons: 1) RP destroys the light detecting cells of the retina but generally leaves the rest of the visual system and body unharmed, so the clinical goal is circumscribed; 2) RP is prototypical of degenerations of the nervous system, so a cure for this less common disease would accelerate progress towards new therapies for a range of more familiar conditions; 3) scientific research has shown that the rods and cones can be spared in animals by either delivering molecules known as growth factors or by transplanting particular types of stem cells, so the scientific feasibility of treating RP has already been established in principle.

The therapeutic approach is to save the light sensing cells of the eye using modified stem cells. The problem with the molecules ("growth factors") that are capable of saving the light detecting cells (rods and cones) is that they are rapidly degraded in the body, so a method of long term delivery is needed. Stem cells can be genetically modified to manufacture and secrete the desired growth factors and thereby save the rods and cones, following transplantation to the eye. In addition, there is evidence that the stem cells themselves might help preserve the cone photoreceptors. Furthermore, recent work has shown that stem cells can to some extent develop into photoreceptors and directly replace the dying cells of the patient's retina. Thus, transplanted stem cells could treat the targeted disease of RP in multiple ways simultaneously. Importantly, there are a host of reasons why clinical trials in the eye are easier and safer than most locations in the body. The eye is an important proving ground for stem cell-based therapies and provides a stepping stone to many incurable diseases of the brain and spinal cord.

**Statement of Benefit to
California:**

Benefits to the state of California and its citizens are both direct and indirect. The direct benefit is medical in that there is currently no cure or established treatment for the individuals and families that suffer from the dreadful hereditary blindness known as retinitis pigmentosa. In addition, there are many people in California and throughout the world that suffer from degenerative diseases of the retina and central nervous system that could benefit from the type of stem cell therapy proposed in the current application. The rapid progress that could be achieved via this proposal would help legitimize the use of stem cells and should thereby accelerate the development of stem cell-based therapeutics for a wide range of other diseases. In so doing there would be an indirect benefit to California by making our state a focal point for stem cell breakthroughs. This would increase medical capabilities, strengthen the [REDACTED] system, and energize local biotechnology companies with outside investment and a payoff in jobs and tax revenues.

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